Application Number: T3-00004 **Scientific Score**: 60 or below

Title: [redacted] Stem Cell Research Training Program

Specific names of individuals and institutions are blacked out to preserve applicant confidentiality where possible.

Proposal Abstract as Submitted by Applicant

[redacted] proposes a CIRM type III program to train Clinical and Post-Doctoral Fellows in stem cell research, with particular emphasis on translational research directed toward pediatric diseases. This program is designed to provide opportunities for Post-Doctoral and Clinical Fellows training for careers in basic, translational, and clinical stem cell research. We are requesting funding to support four (two Clinical and two Post-Doctoral) CIRM Fellows annually. The curriculum will consist of courses and practical, hands-on instruction in stem cell biology and laboratory research. The year-long course work, conducted at the nearby [redacted], will include these three new courses: Basic Biology of Stem Cells; Clinical Applications of Stem Cells; and Social, Legal and Ethical Implications of Stem Cell Research. In addition, CIRM Fellows will be expected to take the Human Embryonic Stem Cell Culture Training Course, of which the Program Director is PI.

As the regional, tertiary-care, children's hospital, [redacted] patients comprise the entire range of pediatric diseases. [redacted] scientists and clinicians currently direct a stem cell research and teaching enterprise working towards therapies for neurometabolic, retinal, immunologic, and hematologic disorders. Specifically, our therapy-directed stem cell research is focused on: lysosomal storage disorders such as Hurler's disease, which we already successfully treat with stem cell transplantation, and the childhood hematologic diseases such as leukemias and dysmyelopoeises including aplastic anemia and congenital marrow failure. We have been successfully treating these latter syndromes for many years with bone marrow, cord blood, and peripheral blood stem cells. Additionally, we have an active stem cell research program directed toward eye diseases such as Kearns-Sayre Syndrome and other pediatric mitochondrial disorders. All of these diseases represent significant causes of mortality and morbidity in the pediatric population and all are potential targets for stem cell therapy.

[redacted] maintains an active academic involvement through [redacted], the Program Director, and [redacted], one of the Mentors, both of whom have academic appointments with [redacted], and [redacted], the Program Clinical Co-Director, who is a professor in Pediatric Hematology/Oncology at [redacted]. This involvement comprises several ongoing and active research collaborations, providing additional research opportunities for CIRM Fellows. These opportunities include research in biomedical engineering, tissue engineering, and genomic replacement.

Benefit of this Program to California

This program will benefit the people and the state of California by providing high-quality training in the scientific, clinical, social, and ethical aspects of stem cell research to the scientists and clinicians who will develop and apply future therapies in this rapidly emerging field.

T3-00004

Summary of Review

This application proposes a type III program to train 2 clinical and 2 post-doctoral fellows in stem cell research, with emphasis on translational research directed toward pediatric diseases. The research program is very focused on the clinical use of stem and progenitor cells but only proposes a couple of projects so it is somewhat limited. The program director is interested in human neural stem cells and directs a National Human Neural Stem Cell Resource based at this institution, but is rather junior and does not have a strong publication record or NIH-supported research funding. The clinical co-director is a well-funded pediatric hematologist who directs hemostasis and thrombosis research. Although the training faculty includes eight potential members, only three are listed as independent mentors. Several have various service responsibilities at this institution and it is unclear how active their laboratory-based research is currently. The applicant pool is limited to clinical fellows selected from residents within the institution's clinical training program. Little information is provided as to the qualifications of these individuals. The institution presents a considerable clinical effort with laboratory support in stem cell research and an active clinical transplant program, which provides the environment for the potential application of stem cell therapeutics. However, it is unclear how these clinical resources will be utilized to facilitate the research training of CIRM Scholars.

Overall Strengths and Weaknesses

The strength of this program is in the emphasis on translational use of stem cells in several specific pediatric disease areas. However it is unclear how much integration between basic science and medical applications will occur. The program lacks a strong research environment that would complement the clinical component at this institution and lacks an adequate pool of mentors for trainees. None of the mentors have NIH funding for their research and few have strong publication records. The applicant pool for fellows seems to be limited, and the pool for post-doctoral fellows was not described.

Recommendations

Not recommended for funding at this time.

	Pre	Post	Clinical	Total
Fellows Requested:	0	2	2	4
Fellows Recommended:	0	0	0	0

	Year 1	Total
Budget Requested:	$ 370,590	$ 1,111,770
Budget Recommended:	0	0